Generic Questionnaire for Biopharmaceutical Production Candidates Involving Recombinant Organisms

Principal Investigator:
Institution:

1. What amount(s) of delivered product(s) is desired?

   A. Non-GMP (laboratory grade)
   B. cGMP (clinical grade)

2. Please provide details/references of your construct or starting materials.

3. If your construct contains an antibiotic-resistance gene please indicate which one? Is this gene used as a selection marker? Are alternative methods of selection available?

4. What other expression systems have been tried for this product?

5. Have you sequenced your construct and if so, is the sequence available in an electronic format?

6. Do you have data evaluating the genetic stability of the recombinant? Have you established mutation rates and or rates of reversion to wild type?

7. Do you have data evaluating the potential for genetic recombination or plasmid transfer with other organisms in the patient or in the environment? Please describe.

8. Is the organism currently being grown in a qualified cGMP cell line? If not, is there a qualified cell line available for propagation of this organism?

9. Do you have a complete history of the cell line or clone you are currently using?

10. Do you have a Cell Bank?

11. Please provide details of your production method.

12. Has this material ever been produced for laboratory or clinical studies using this production system?

13. Has this material ever been produced in a related or other production system? If so please provide details.

14. Please provide details of your purification methods.

15. What is the average yield of your expression system before and after purification? What is the largest amount of material that you have produced in your laboratory in a single

production batch?

16. Do you have any material to supply as a reference standard?

17. Do you have any material to supply as bulk drug for preliminary pharmacology and toxicology studies? If so, how much is available for these studies?

18. Do you have reproducible assays for your product? Please describe the following assays for evaluating your material, if available:

    Identity:
    Purity:
    Potency:

19. Do you have a proposed list of release criteria for your product? If so, please provide specifics.

20. What is known about the stability of your product with respect to physical integrity and activity?

21. In what form and fill size do you want the final product?

22. Are there issues of formulation that must be resolved?

23. Do you have any information regarding the estimated costs associated with this project?

24. Have you identified any possible sources of production with any commercial firms? Please provide any details that you have.

25. Are there any safety issues connected with the production, purification, and/or handling of your product?

26. What is the status of your product(s) regarding intellectual property issues?

27. Sometimes, proposed projects are an improvement or modification of an existing approach. In these cases, this information may affect significantly affect our analysis of feasibility, cost, and other production issues. Of course, this information may also be important in consideration of intellectual property issues. To the extent that you are aware, please provide a brief summary of the nature of any such antecedents or other approaches that may appear closely related to the project you propose.